香港罕見疾病聯盟
RARE DISEASE HONG KONG

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Submission on the Public Consultation for the 2021 Policy Address
(September 2021)

Established in December 2014, Rare Disease Hong Kong (RDHK) is the first patient group in Hong Kong comprising cross-rare-disease patients and their families with the support of experts and academics in the field. Representing the patients and caregivers, RDHK is committed to enhancing public awareness of rare diseases in collaboration with the stakeholders. We aim to improve rare disease policies and services, and to ensure equal respect and protection for patients in terms of fundamental rights such as healthcare, social support, education and daily needs.

Since the current-term Government took office, new measures on rare diseases have been proposed in every policy address, including increasing the Safety Net support for orphan drugs, optimizing the Safety Net means test mechanism, establishing patient database, conducting public education, increasing manpower, supporting scientific research and clinical research, etc., which have been or are being implemented in phases. RDHK highly appreciates all such initiatives which respond positively to the aspirations of the patient community.

The last policy address of the current-term Government will be delivered in October this year. It would therefore be a suitable time to review the Government's achievements and shortcomings over the past four years so that governance of the new term Government can be more effective.

The new measures on rare diseases proposed by the current-term Government in the previous years can certainly help to address and solve the specific problems of individual diseases. However, these measures are mostly sporadic, fragmented, unsystematic and largely reliant on clinical means only, which is far from satisfactory in terms of effectiveness of tackling rare diseases comprehensively. Expecting that the new policy address can continue with past successes while creating new achievements in the future, RDHK hopes the Government will adopt a macro perspective and foreword-thinking approach to policy development so that more systematic, comprehensive and integrated measures can be launched in phases. RDHK would like to propose **the basic framework** and **short-term measures** as follows:

Basic framework:

I. To set up the "Steering Committee on Strategy for Rare Diseases" with tripartite collaboration among the government, the business sector and the community.

In recent years, the local community has become increasingly concerned about rare diseases. The introduction of new measures by the Government in consequent years has contributed to fostering the interest and investment in rare diseases by universities, scientific research institutions, technology enterprises, healthcare and pharmaceutical industries, etc.; the ever-growing experience of the HA staff including the management and frontline professionals in screening, diagnosing and treating rare diseases; and a more comprehensive and in-depth understanding of the rare disease issues among the government officials. Building on the current basis, more passion and strengths can be gathered, and professional knowledge and resources can be effectively coordinated. To maximise the outcomes of tripartite

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collaboration among the government, the business sector and the community with minimal effort, the Government with an executive-led system must inevitably play a leading role.

Therefore, RDHK suggests that the "Steering Committee on Strategy for Rare Diseases" should be formed with the Secretary for Food and Health as chairperson, and the Steering Committee should include government officials, rare disease experts and representatives of the Hospital Authority (HA), two medical faculties, patient groups and the business sector. The core responsibilities of the Steering Committee are to take reference from the experience of Europe, the United States and neighbouring countries and regions (such as the Mainland, Taiwan, Australia, etc.) so as to advise the Chief Executive on the formulation and phased implementation of the "Hong Kong Rare Disease Action Plan" in accordance with the situation in Hong Kong and pursuing what have been practising in the past few years; and to co-ordinate the implementation of the rare disease measures proposed by the Chief Executive, monitor the progress of implementation and report to the Chief Executive. If necessary, the Steering Committee may set up sub-committees or working groups with different responsibilities and functions to bring together experts from the Government and the private sector to tackle the challenges of rare diseases together.

II. To define "rare diseases" and compile the rare disease list

There has been no official definition of rare diseases in Hong Kong. In recent years, the term "uncommon disorder" has been used in government official documents. However, the Food and Health Bureau (FHB) has so far failed to define or give any example of "uncommon disorder". It is hard to understand why the Government still adopts such an evasive and vague attitude towards this issue. Even though the policy documents have been in place for several years, we only know the term "uncommon disorder" by name. Consequently, different stakeholders have their own definition of rare diseases. Knowing that it is not that easy to work out a definition which is scientifically acceptable and complies with the situation in Hong Kong, RDHK does not expect the Government to finish the job overnight. However, it is always inescapable for the Government to formulate clinical and medical definitions if it is sincere and determined to meet the challenges of rare diseases. RDHK urges the Government to seize the opportunity of setting up the "Steering Committee on Strategy for Rare Diseases" to deal with the important task of defining rare diseases in a local context.

Meanwhile, the Government can take the first step in compiling the rare disease list by recognising the 40 "uncommon disorders" or indications that are treated with special drugs, drugs supported by the Care Community Fund, Samaritan Fund and some compassionate programmes as listed in the Drug Formulary. In the future, more rare diseases can be added to the list after the official definition of rare diseases has been established, which can facilitate compliance by all stakeholders.

<u>Short-term measures</u>:

I. To speed up the process of introduction of new drugs

At present, the registration process for orphan drugs in Hong Kong is the same as that for general drugs, i.e. two certificates of pharmaceutical product (CPP) issued by the U.S. Food and Drug Administration (FDA), the European Medicines Agency (EMA), or any drug regulatory authorities in Australia, Canada,

Japan, etc. are required. Taking reference from other regions around the world (including Mainland China), orphan drugs are assessed separately from general drugs to speed up the approval process. At a meeting of the Legislative Council Panel on Health Services this year, a Legislative Councillor suggested that orphan drugs approved by one of the drug regulatory authorities in the US or Europe can be registered in Hong Kong. Based on the track record of the safety and efficacy of drugs approved by the US and European drug regulatory authorities, RDHK strongly supports the above suggestion in the hope of speeding up the process of orphan drug registration in Hong Kong.

II. To adopt "risk sharing" arrangements, and to speed up the inclusion of orphan drugs in the Safety Net by providing clear guidelines on the review mechanism

Although the HA has introduced some expensive orphan drugs in recent years, there is still a lack of a clear review procedure, which makes the clinical experts, patients and pharmaceutical industry all at a loss. In fact, it is doubtless that the safety and efficacy of drugs registered in Hong Kong are all recognised by the drug regulatory authorities in Europe and the US; whereas the availability of local data depends on the number of clinical applications but such data can never be collected if a drug is not used. As regards the so-called "cost-effectiveness", it is learned that the HA and FHB actually do not have health economics experts. So, focus is placed on cost only while other essential factors like healthcare efficacy and social impacts are not scientifically evaluated. RDHK recommends that more scientific health economic analysis such as "patient-reported outcomes" and "risk-sharing" arrangements should be adopted in the review mechanism of orphan drugs, based on which clear guidelines can be laid down for the clinical physicians, patients, pharmaceutical companies and decision makers. As a number of orphan drugs were approved for use in the past few years, it is believed that the clinical experts and management had gained experience in approving orphan drugs, which to some extent should lay the foundation for the development of clear selection guidelines.

III. To enrich the rare disease database

To implement the initiative of establishing a database of rare diseases as listed in the 2019 policy address, HA has embarked on the first stage of work, covering more than 20 rare metabolic disorders and spinal muscular atrophy. RDHK requests that more rare diseases should be included in the database platform in the next two to three years to make the data meaningful in clinical, research and public health decision making. Apart from the HA-led database on rare diseases, some clinical experts and academics have set up databases on individual diseases in recent years, such as the Hong Kong Neuromuscular Disorders Patient Registry managed by the University of Hong Kong, Tuberous Sclerosis Complex Patient Registry, Registry of Hereditary Retinal Diseases, and so on. In planning to expand the database on rare diseases, the Government should first learn more about the existing projects in the community and make the best possible use of and coordinate available resources in order to achieve compatibility and convergence.

IV. To expand the scope of newborn screening for rare diseases

The "Newborn Screening Programme for Inborn Errors of Metabolism (IEM)" was launched as a pilot scheme in 2015. It has now been extended to eight public hospitals covering 26 IEM conditions. As more treatments for individual rare diseases become available, it is necessary for the Government to regularly

review and include new rare diseases in the scope of newborn screening basing on the principle for selection of condition for screening published by World Health Organization, such as "whether facilities for diagnosis are available", "whether there is accepted treatment for patients with recognised disease", "the prevalence of the disease", "the benefits of early diagnosis and treatment for patients", and so on.

V. To promote and develop one-stop consultation service with a view to gaining experience in establishing the "Centre of Excellence for Rare Diseases"

At present, the HA provides medical consultation to rare disease patients according to its administrative arrangements without taking the conditions of patients and medical efficacy into account. Some patients have to visit multiple specialties, but the individual specialty doctors only focus on their own specialty area, and therefore may not be able to get the whole picture of a patient's condition. Such practice has been widely criticised because both doctors and patients have put in a lot of time and efforts, but with little effect. With expert support, the Hong Kong Children's Hospital (HKCH) has rolled out the one-stop consultation service for child patients with tuberous sclerosis complex (TSC) this year, which is a huge breakthrough. In fact, HKCH was established to focus on dealing with pediatric diseases that are serious, complex, uncommon and require cross-specialty care, so it has got what it takes to set up the prototype of "Centre of Excellence for Rare Diseases". RDHK expects that the one-stop service for TSC patients is a precedent for other rare diseases in HKCH. Furthermore, it is hoped that one-stop consultation service will be extended to adult patients with rare diseases and all the cases of rare diseases can be treated in individual hospitals as far as possible. This can pave the way and gather experience for the establishment of a territory-wide "Centre of Excellence for Rare Diseases" in the future.

Conclusion

The goal of Hong Kong's public health policy is "to ensure that that no one is denied adequate medical care due to lack of means". To provide "adequate medical care" for patients with rare diseases, we must first acknowledge and tackle the differences between rare diseases and common diseases by adopting a humanised approach, which takes diversity, equity and inclusiveness into account. The conventional healthcare policies and measures can only respond to the needs of ordinary patients; but are unsuitable and inadequate on multiple fronts for the rare disease patients with special needs. The Government must change its traditional administrative mindset, make good use of the community wisdom and resources, draw on overseas experience, and ensure the relevance of policies and enhance the effectiveness of governance through tripartite collaboration among the Government, the business sector and the community.

The political environment in Hong Kong is getting stable, which provides an unprecedented opportunity for the Government to push forward with economic development and improve peoples livelihood, including care for the rare disease patients. RDHK expects that a responsible Government should always upholds the belief that the people and their lives come first, and will make commitment to the hundreds of thousands of rare disease patients.

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